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Creating Decision Trees to Assess Cost-Effectiveness in Clinical Research

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Abstract

Decision analysis modeling has emerged as a powerful tool to weigh the cost-effectiveness of complex healthcare decisions. Decision analysis utilizes mathematical models to quantitatively compare multiple decisions accounting for both the monetary cost and the effect on quality of life. The current article reviews the components, statistical analyses, strengths, and limitations of decision analysis modeling for cost-effectiveness research in medicine.

Keywords: Decision analysis model; Cost-effectiveness; Quality of life years; Utilities

Introduction

In 2009 the average per capital National Health Expenditure (NHE) was $8,086, or 17.6% of the Gross Domestic Product (GDP), a 4% increase from merely one year earlier [1]. As healthcare costs soar at unprecedented rates, physician, economists and law makers are increasingly faced with evaluating the cost-effectiveness of different practice patterns. Cost-effectiveness encompasses not only the monetary cost of a test or drug but also considers the cost of making an alternate decision and the effect the decision will have upon quality of life. Decision analysis modeling uses existing evidence to create a mathematical model that evaluates the consequences of a particular decision [2]. When decision analysis modeling is applied to healthcare decision making it becomes a very powerful tool to evaluate cost-effectiveness. Decision analysis models have been used to compare screening strategies, diagnostic techniques and treatment plans. As cost-effectiveness gains increasing importance, decision analysis modeling is likely to take a more prominent role in biomedical research.

Building a Cost-Effectiveness Decision Analysis Model

A decision analysis model is comprised of multiple components. Careful attention to each component yields the most reliable model incorporating all the critical variables and effects.

Decision

The first, and most critical, component is the clinical decision. The study question can encompass any medical practice but most often centers on the cost-effectiveness of different screening or diagnostic tools or the choice between treatment options. The flexibility of decision analysis allows for evaluation of a single strategy choice compared with no intervention or multiple strategies may be weighed. In recent years, decision analysis has been utilized to evaluate the cost-effectiveness of many clinical options such as HIV screening, sentinel lymph node biopsy in breast cancer patients and laparoscopic vs. hysteroscopic sterilization [3-5].

Consequences and probability of consequences

After establishing the clinical question, one must evaluate the consequences of each competing clinical decision. It is important to consider both intended and unintended consequences. Additionally one must consider the probability of each consequence. This information is typically gleaned from an extensive literature search. Meta analyses and randomized controlled trials are ideally used but cohort studies, case controls and expert opinions can also be used to identify the consequences of differing decisions and the likelihood of those consequences. Opposing decisions, their effects and consequences can be organized on a tree graph.

Cost of decision and consequence

An extensive literature search can also yield important insight into the monetary cost of each competing decisions and the financial cost associated with the consequences of the competing decisions. When comparing financial cost, it is critical to adjust to a single currency and year to ensure equivalent evaluation. Costs can be adjusted by year using the Consumer Price Index. On average, costs are discounted by three percent per year.

Estimating effect

The final component of the decision analysis model requires estimating the effect on quality of life. Quality Adjusted Life Years (QALY) is now the standard measure which encapsulates both improvement in the length and quality of life [6]. The quality of each year of life is referred to as the utility and given a numerical value. The values are typically obtained from the literature and based on large cohort surveys. Utilities are then summed over a lifetime to obtain QALY. As current quality of life is often valued over future quality, QALYs are also discounted like costs, typically 3% per year with a range of 1 to 5%.

Cost calculation

Once all of the components of the model have been gathered, the decision analysis can be performed using mathematical equations. The total adjusted cost of any single decision is simply the monetary cost of each consequences multiplied by the probability of that consequence. Each probability and consequence is added to yield a total cost (Figure 1). The difference between the costs of two different strategies is then divided by the difference between the total effects (QALYs) of the two strategies. This yields the Incremental Cost-Effectiveness Ratio (ICER), the primary outcome for most cost-effectiveness decision analyses. A variety of software packages are currently available which calculate

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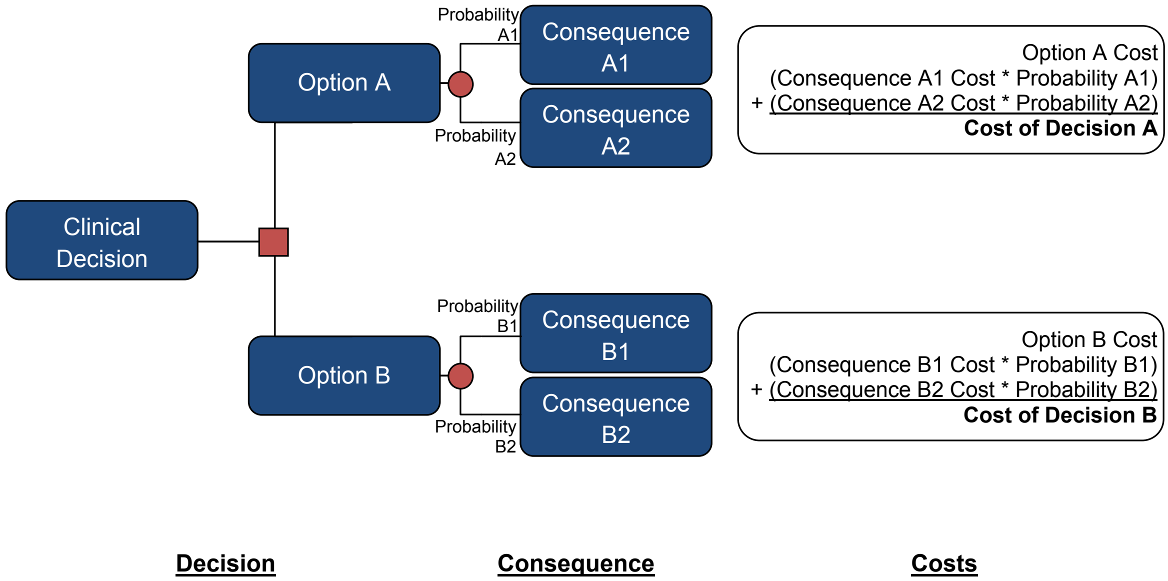

Figure 1: Calculating Cost Using a Decision Tree.

ICER based on these formulas. Tree Age Pro (Willliamstown, MA) and Decision Tools Suite (Ithaca, NY) are examples of commonly utilized decision analysis modeling software.

Results

To ultimately decide which option is more cost-effective, one must establish a point at which the cost differential makes one decision truly superior to another. Historically, this threshold was linked to the cost of dialysis per year and was approximately $50,000 per QALY gained [7]. More recently, however, studies are citing $100,000 per QALY gained as an acceptable Willingness To Pay (WTP) threshold although the WTP depends on the clinical situation, location and population.

Sensitivity analysis

It is often impossible to assign a single dollar value to an individual consequence. Costs often vary with location, season and multiple other factors. Sensitivity analyses allow the investigator to vary a probability, cost or quality variables within the model while keeping all other probabilities, costs and qualities stable. This allows the investigator to explore the cost-effectiveness if each variable is stretched to its plausible extremes. One-way, two-way and three-way sensitivity analyses are often reported based on how many variables are allowed to fluctuate at a time; one, two or three respectively. These analyses serve as an alternative to confidence intervals which do not have value in decision models. Monte Carlo analyses are the extreme example of sensitivity analyses in which all variables are allowed to simultaneously vary to their extremes.

Limitations of Decision Analysis

Although decision analysis modeling is a powerful research tool, several limitations must be recognized. First, and foremost, decision analysis modeling is dependent upon the quality of the data points acquired from the literature search and background data gathering. Decision analysis is reliant upon rigorous research that proposes interesting clinical questions along with thoughtful consideration of the effects of each decision. Without careful consideration of all the consequences of each decision the results from decision analysis will yield an oversimplified result not accounting for all direct costs or consequences. Additionally, decision analysis rarely if ever accounts completely for indirect costs and consequences.

Quality of life estimation is equally fraught with possible error. It is impossible to capture such a qualitative phenomenon that incorporates personal, cultural and psychological beliefs that are very among individual within a single numerical value [6]. Although utilities are well studied and are a verified method of quality of life assessment, it remains an imperfect method of quantifying such a qualitative outcome.

Strengths of Decision Analysis

Despite its limitations, decision analysis modeling is a necessary tool for clinicians, researchers and law-makers who are increasingly faced with ballooning health care costs. In addition to yielding invaluable information, decision analysis modeling is faster than traditional research techniques, associated with minimal cost and requires no direct patient risk. As cost-effectiveness gains increasing importance in medical practice patterns, decision analysis is likely to take a more prominent role in biomedical research.

Conclusion

Between 1999 and 2008 the FDA approved 259 new drugs for use in the United States [8]. While the rate of innovation in pharmaceutical and diagnostic therapies provides hope for the ailing, with each new treatment comes a cost. Comprehensive patient care requires that the benefits of new and existing therapies be weighed against their costs. Decision analysis modeling is a key piece of the armamentarium for healthcare decision makers struggling to balance providing the highest quality of care with sky rocketing costs.

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